CIRM Board Approves New Clinical Trial for Rare Childhood Disease

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Oakland, CA – Today the governing Board of the California Institute for Regenerative Medicine (CIRM) approved a grant of almost $12 million to Dr. Stephanie Cherqui at the University of California, San Diego (UCSD) to conduct a clinical trial for treatment of cystinosis.

This award brings the total number of CIRM funded clinical trials to 55.

Cystinosis is a rare disease that primarily affects children and young adults, and leads to premature death, usually in early adulthood. Patients inherit defective copies of a gene called CTNS, which results in abnormal accumulation of an amino acid called cystine in all cells of the body. This buildup of cystine can lead to multi-organ failure, with some of earliest and most pronounced effects on the kidneys, eyes, thyroid, muscle, and pancreas. Many patients suffer end-stage kidney failure and severe vision defects in childhood, and as they get older, they are at increased risk for heart disease, diabetes, bone defects, and neuromuscular defects. There is currently a drug treatment for cystinosis, but it only delays the progression of the disease, has severe side effects and is expensive.

Dr. Cherqui's clinical trial will use a gene therapy approach to modify a patient's own blood stem cells with a functional version of the defective CTNS gene. Based on pre-clinical data, the approach is to reintroduce the corrected stem cells into the patient to give rise to blood cells that will reduce cystine buildup in affected tissues.

Because this is the first time this approach has been tested in patients, the primary goal of the clinical trial is to see if the treatment is safe. In addition, patients will be monitored for improvements in the symptoms of their disease. This award is in collaboration with the University of California, Los Angeles which will handle the manufacturing of the therapy.

CIRM has also funded the preclinical work for this study, which involved completing the testing needed to apply to the Food and Drug Administration (FDA) for permission to start a clinical trial in people.

"CIRM has funded 24 clinical stage programs utilizing cell and gene medicine approaches to date," says Maria T. Millan, M.D., the President and CEO of CIRM. "This project continues to broaden the scope of unmet medical need we can impact with these types of approaches."

About CIRM

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet medical needs, and act with a sense of urgency to succeed in that mission.

To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today's most promising stem cell technologies.

With $3 billion in funding and approximately 300 active stem cell programs in our portfolio, CIRM is the world's largest institution dedicated to helping people by bringing the future of cellular medicine closer to reality.

For more information go to www.cirm.ca.gov